FOOD AND DRUG ADMINISTRATION (FDA)

Center for Drug Evaluation and Research (CDER)

Peripheral and Central Nervous System Drugs Advisory Committee Meeting

The Inn and Conference Center, University of Maryland University College (UMUC)
Marriott Conference Centers
3501 University Boulevard East, Adelphi, MD

QUESTIONS TO THE ADVISORY COMMITTEE

MAY 6, 2010

1. Substantial evidence of effectiveness can consist of data from adequate and well-controlled clinical investigations (replication) or a single adequate and well controlled clinical investigation and confirmatory evidence.

    a. Has the sponsor provided substantial evidence of effectiveness for Acthar Gel as a treatment for patients with Infantile Spasms (IS)? **[Voting Question]**

    b. If so, which standard described above has been met?

2. If the answer to Question 1 is yes, has effectiveness been shown in: 1) cessation of spasms, 2) amelioration of the EEG, 3) prevention of other seizure types, 4) improvement in long-term developmental outcomes, or 5) other outcomes?

3. If the answer to Question 1 is no, what additional data should be obtained prior to approval?

4. The sponsor wishes to recommend a 2 week course of treatment, followed by a 2 week tapering regimen. Has the sponsor submitted evidence to support the view that a short course of treatment provides sustained effectiveness? **[Voting Question]**

5. If the answer to Question 4 is no, can you provide dosing recommendations (for example, is there evidence that continued treatment beyond 2 weeks is appropriate, or is there evidence that repeated, intermittent short courses of treatment are useful)?

6. Acthar Gel has been shown to cause serious adverse effects, and the sponsor concludes that they are predictable, easily recognized and manageable, and reversible upon drug discontinuation.

    Has the sponsor provided evidence that the adverse events are manageable and reversible? **[Voting Question]**

7. Has the sponsor submitted sufficient evidence of the safety of Acthar Gel at an effective dosing regimen? **[Voting Question]**

8. If the answer to Question 7 is no, what additional safety data should be obtained prior to approval?

9. Are there patients in whom Acthar Gel should be contraindicated (e.g., infants with hypertension, infections, or metabolic disorders)?

10. Does the committee recommend any specific monitoring for specific adverse events?

    If so, should these be made mandatory under a REMS?